

20 January 2012

## Proposal to widen access to ursodeoxycholic acid

PHARMAC is seeking feedback on a proposal to widen funded access to ursodeoxycholic acid for prevention of veno-occlusive disease in patients receiving high dose conditioning therapy prior to bone marrow or stem cell transplantation.

Further details of this proposal, including how to provide feedback and background information, can be found below and on the following pages.

### Feedback sought

PHARMAC welcomes feedback on this proposal. To provide feedback, please submit it in writing by **5 pm Tuesday the 7<sup>th</sup> of February 2012** to:

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All feedback received before the closing date will be considered by PHARMAC's Board (or Acting Chief Executive acting under delegated authority) prior to making a decision on this proposal.

### Details of the proposal

Ursodeoxycholic acid is listed in Section B of the Pharmaceutical Schedule under Special Authority restriction for use in cholestasis of pregnancy or cirrhosis. PHARMAC is proposing to amend the Special Authority Criteria that apply to ursodeoxycholic acid from 1 March 2012, as follows (additions in bold):

Initial application – **(Pregnancy/Cirrhosis)** - from any relevant practitioner. Approvals valid for 6 months for applications meeting the following criteria:

Either:

1. Patient diagnosed with cholestasis of pregnancy; or
2. Both:
  - 2.1. Primary biliary cirrhosis confirmed by antimitochondrial antibody titre (AMA) > 1:80, and raised cholestatic liver enzymes with or without raised serum IgM or, if AMA is negative, by liver biopsy; and
  - 2.2. Patient not requiring a liver transplant (bilirubin > 170umol/l; decompensated cirrhosis)

Note: Liver biopsy is not usually required for diagnosis but is helpful to stage the disease.

**Initial application – (Haematological Transplant) - from any relevant practitioner. Approvals valid for 4 months for applications meeting the following criteria:**

**Both:**

- 1. Patient at risk of veno-occlusive disease or has hepatic impairment and is undergoing conditioning treatment prior to allogenic stem cell or bone marrow transplantation, and**
- 2. Treatment for up to 13 weeks.**

**Renewal – (Pregnancy/Cirrhosis) - from any relevant practitioner. Approvals valid for 2 years where the treatment remains appropriate and the patient is benefiting from treatment.**

Note: Ursodeoxycholic acid is not an appropriate therapy for patients requiring a liver transplant (bilirubin > 170 micromol/l; decompensated cirrhosis). These patients should be referred to an appropriate transplant centre. Treatment failure – doubling of serum bilirubin levels, absence of a significant decrease in ALP or ALT and AST, development of varices, ascites or encephalopathy, marked worsening of pruritus or fatigue, histological progression by two stages, or to cirrhosis, need for transplantation.

## **Background**

PHARMAC has received a number of applications for ursodeoxycholic acid through the Hospital Exceptional Circumstances (HEC) scheme for use as part of conditioning therapy in stem cell or bone marrow transplant recipients to prevent veno-occlusive disease. In addition, there has been a recent price increase for the alternative treatment in this setting, defibrotide and using ursodeoxycholic acid would be cost saving to DHBs.

Clinical advice on the funding of ursodeoxycholic acid was sought from the Cancer Treatments Subcommittee at its April 2011 meeting. The relevant excerpt from the minutes of that meeting is as follows:

*The Subcommittee noted that PHARMAC had received a number of Hospital Exceptional Circumstances applications for ursodeoxycholic acid for the prevention of hepatic complications and veno-occlusive disease in patients receiving high dose conditioning therapy prior to bone marrow or stem cell transplantation. Members noted that defibrotide used to be used for this indication but its price had recently increased significantly and therefore hospitals were switching to ursodeoxycholic acid as a partial alternative as it was cost saving.*

*The Subcommittee recommended that the Special Authority criteria applying to the funding of ursodeoxycholic acid in Section B of the Pharmaceutical Schedule be amended to include funding for up to 13 weeks for patients at risk of veno-occlusive disease, or those with hepatic impairment, undergoing intensive conditioning treatment prior to allogeneic stem cell or bone marrow transplant.*

*The Subcommittee recommended that the Special Authority not be removed from ursodeoxycholic acid until consideration were given to the fiscal risk from its increased use as chemoprevention for colorectal cancer in patients with ulcerative colitis.*

Amending the Special Authority criteria as proposed would enable patients undergoing a haematological transplant, who are at risk of veno-occlusive disease, to receive appropriate prophylactic treatment in a timely fashion.

We are not proposing to remove the Special Authority restriction entirely from ursodeoxycholic acid at this time. However, we intend for the Gastrointestinal Subcommittee to review these criteria at its next meeting in March 2012 and to provide advice on any further patient groups who may benefit from treatment.